New disease-specific embryonic stem cell lines from Michigan

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Stem cell scientists at the University of Michigan and in Detroit have created two embryonic stem cell lines that contain disease-causing mutations: Hemophilia B, a hereditary condition in which the blood does not clot properly and Charcot-Marie-Tooth disease, an inherited disorder leading to degeneration of muscles in the foot, lower leg and hand.

For the first time, scientists will have a way of studying cells that carry the causing mutation and understanding how the disease arises. When the mutation is in embryonic stem cells, it is then carried by any cell type emerging from that line. Maturing the hemophilia line into blood cells, for example, could provide insights into genetic factors associated with disease. These cells also provide a way to test possible therapies in human cells rather than in animals that mimic the disease.

The cells came from embryos created through in vitro fertilization that were determined by preimplantation genetic testing to carry a disease mutation. A few cells from the 3-5 day old IVF embryo are sent to the clinic, and the parents can choose which embryos to implant based on the results. Embryos with possibly lethal disease mutations are generally destroyed as medical waste. Donating t research gives couples an option other than simply destroying the embryos.

The Detroit News wrote about the new lines:

" U-M will soon be submitting these disease-specific lines to the National Institutes of Health to be placed on the Human Embryonic Stem Cell Registry. Researchers across the country will be able to use the lines for federally funded research. Of the 91 lines currently on the registry, three are disease-specific stem cell lines submitted by Harvard and Stanford universities.
In the story, Bernard Seigal, executive director of the Florida-based Genetics Policy Institute that hosts the World Stem Cell Summit (to be co-hosted this year by CIRM) said this discovery is a direct result of the passage of Proposal 2, a constitutional amendment that allowed for embryonic stem cell research in Michigan.

" The passage of Proposal 2 wasn't just a political statement," Siegel said. "This has been followed up with real, tangible research and real results that have the potential to impact human health. It portends very well for the future of stem cell research in Michigan."
CIRM funds several awards to grantees who are developing embryonic stem cell lines that were found to carry disease-causing mutations through preimplantation genetic testing. These include Julie Baker at Stanford University and Amander Clark at UCL.

- A.A.

Tags: Clark, University of California Los Angeles, Stanford University, baker, preimplantation genetic testing, hemophilia, IVF